(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization

International Bureau



(43) International Publication Date 28 July 2005 (28.07.2005)

PCT

(10) International Publication Number WO 2005/067632 A2

(51) International Patent Classification:

(21) International Application Number:

PCT/US2005/000418

Not classified

(22) International Filing Date: 7 January 2005 (07.01.2005)

(25) Filing Language:

English

(26) Publication Language:

English

US

US

US

(30) Priority Data:

7 January 2004 (07.01.2004) 60/535,042 60/556,843 27 March 2004 (27.03.2004) 60/557,232 29 March 2004 (29.03.2004)

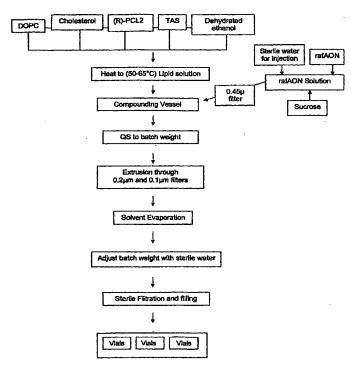
(71) Applicant (for all designated States except US): NEOPHARM, INC. [US/US]; 150 Field Drive, Suite 195, Lake Forest, Illinois 60045 (US).

(72) Inventors; and

(75) Inventors/Applicants (for US only): AHMAD, Moghis U. [US/US]; 3050 North Forest Hills Ct., Wadsworth, Illinois 60083 (US). SHEIKH, Saifuddin [US/US]; 1059 South West Avenue, Waukegan, Illinois 60085 (US). ALI, Shoukath [IN/US]; 29681 North Waukegan Road, #204, Lake Bluff, Illinois 60044 (US). CHIEN, Pei-Yu [US/US]; 760 Pleasant Avenue, Highland Park, Illinois 60035 (US). JAMIL, Haris [US/US]; 1216 Trinity Place, Libertyville, Illinois 60048 (US). ZHANG, Jia-Ai [US/US]; 1251 Maidstone, Vernon Hills, Illinois 60061 (US). UGWU, Sydney [NG/US]; 4005 Stoney Island, Gurnee, Illinois 60031 (US). ZHANG, Zhi-Yi [CN/US]; 7986 Dada Drive, Gurnee, Illinois 60031 (US). WANG, Jinkang [US/US]; 39 Inverness Drive, San Francisco, California 94132 (US). BHAMIDIPATI, Shastri [US/US]; 2380 Chambound Drive, Buffalo Grove, Illinois 60089 (US). AHMAD, Zafeer [US/US]; 2933 North Augusta Drive, Wadsworth, Illinois 60083 (US). AHMAD, Imran [US/US]; 4731 West Pebble Beach, Wadsworth, Illinois 60083 (US).

[Continued on next page]

(54) Title: LIPID COMPOSITIONS AND USE THEREOF



(57) Abstract: The invention provides a composition suitable for use as a transfection agent, comprising a cationic cardiolipin analogue and, another lipid species. The composition of the present invention can facilitate transfection of a wide variety of polynucleotide species (e.g., oligodeoxyribonucleotides, plasmids, RNAi species, etc.). Moreover, the transfection agent of the present invention is effective in promoting transfection of primary cell cultures as well as transformed cells. Also, the inventive transfection agent is suitable for use both in vitro and in vivo. The inventive composition has additional uses as well, such as delivery of a variety of active agents, dermatological and cosmetic uses, and uses in agriculture. The invention further. provides a method of introducing an active agent into a cell by contacting the cell with the inventive composition. The invention further provides a method of inhibiting the growth of neoplastic cells and a method of treating a patient suffering from a neoplastic disease by employing the inventive composition, wherein an active agent is an antineoplastic agent. The invention further provides a method for validating a genetic target, comprising (a) administering to a cell a composition comprising a cationic liposome and a siRNA, whereby the siRNA enters the cell inhibits the expression of a gene within the cell and (b)

assaying for the inhibition of the gene. The method also provides a fluorescent/ luminescent cationic cardiolipin analogue and compositions including such analogues. Using a cationic cardiolipin analogue, the invention provides a method of tracking the migration of a lipid substance within an animal.



- (74) Agents: HEFNER, M. Daniel et al., LEYDIG, VOIT & MAYER, LTD., 180 North Stetson Avenue, Two Prudential Plaza, Suite 4900, Chicago, Illinois 60601-6780 (US).
- (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IS, IT, LT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

 without international search report and to be republished upon receipt of that report

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.